Hematopoietic Stem Cell Gene Therapy for IPEX Syndrome

Grant Award Details

Hematopoietic Stem Cell Gene Therapy for IPEX Syndrome

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-12895

Project Objective: Autologous stem cell gene therapy for IPEX syndrome. To perform process development, preclinical safety and efficacy studies to support Pre-IND meeting with the FDA.

Investigator:

Name:	Katelyn Masiuk
Institution:	ImmunoVec
Type:	PI

Disease Focus: Blood Disorders, IPEX Syndrome

Human Stem Cell Use: Adult Stem Cell

Award Value: $3,551,332

Status: Active

Grant Application Details

Application Title: Hematopoietic Stem Cell Gene Therapy for IPEX Syndrome

Public Abstract:

Translational Candidate

Human hematopoietic stem cells that have been modified to express a functional FOXP3 gene to treat patients with IPEX Syndrome

Area of Impact

These studies will bring stem cell gene therapy for IPEX closer to the clinic especially for those without an HLA match or disease too severe for HSCT

Mechanism of Action

Hematopoietic stem cells (HSCs) with defective Foxp3 expression are modified with a lentiviral vector which restores a normal copy of the defective gene. Transplantation of gene-modified HSCs, which are self-renewing and long-lived, produce all blood lineages, including regulatory T cells with restored FoxP3 expression that can control the severe autoimmunity present in IPEX Syndrome

Unmet Medical Need

There is no curative treatment for IPEX patients without a bone marrow match. Gene corrected HSC can cure IPEX and provides a therapeutic option for these patients. This proposal will advance the field of stem cell gene therapy and treatment of primary immune disorders.

Project Objective

Pre-IND meeting

Major Proposed Activities

- Obtain clinical grade lentiviral vector and demonstrate the ability to manufacture the stem cell product at clinical scale
- Perform rodent studies to assess safety and the effective dosage of the cell product
- Prepare clinical protocol, investigator's brochure, consent forms, and Pre-IND package. Complete Pre-IND meeting with the FDA.

Statement of Benefit to California:

Safe, definitive therapies for IPEX Syndrome represent an unmet medical need. Allogeneic stem cell transplant is frequently complicated by graft-versus-host disease or limited by lack of HLA matched donors. Successful demonstration that stem cell gene therapy can safely and effectively cure IPEX will shift the paradigm by which patients will be treated and provide a foundation by which other immune and blood diseases may be cured in the future.
